

## Human iPSC modeling and therapeutics for degenerative peripheral nerve disease

### Grant Award Details

Human iPSC modeling and therapeutics for degenerative peripheral nerve disease

**Grant Type:** New Faculty Physician Scientist

**Grant Number:** RN3-06530

**Project Objective:** To develop human iPSC lines from patients with inherited peripheral neuropathy (Charcot Marie Tooth type 1A, or CMT1A) due to duplication of the PMP22 gene. The PI proposes to genetically correct the defect in these cells, differentiate them into Schwann cell precursors (SCPs), and determine whether genetic correction recovers their ability to effectively myelinate axons in vitro and in vivo. Finally they will transplant genetically corrected SCPs into a rat model of CMT1A, to determine whether these cells protect peripheral axons and alter disease progression.

**Investigator:**

|                     |                             |
|---------------------|-----------------------------|
| <b>Name:</b>        | Robert Baloh                |
| <b>Institution:</b> | Cedars-Sinai Medical Center |
| <b>Type:</b>        | PI                          |

**Disease Focus:** Neurological Disorders, Neuropathy

**Human Stem Cell Use:** iPS Cell

**Cell Line Generation:** iPS Cell

**Award Value:** \$3,031,737

**Status:** Active

### Progress Reports

**Reporting Period:** Year 1

[View Report](#)

**Reporting Period:** Year 2

[View Report](#)

**Reporting Period:** Year 3

[View Report](#)

**Reporting Period:** Year 5

[View Report](#)

---

## Grant Application Details

---

**Application Title:** Human iPSC modeling and therapeutics for degenerative peripheral nerve disease

**Public Abstract:** The applicant is an MD/PhD trained physician scientist, whose clinical expertise is neuromuscular disorders including peripheral nerve disease. The proposal is aimed at providing a research proposal and career development plan that will allow the applicant to develop an independent research program, which attempts to bring stem cell based therapies to patients with peripheral nerve diseases. The proposal will use "adult stem cells" derived from patients with an inherited nerve disease, correct the genetic abnormality in those cells, and determine the feasibility of transplanting the genetically engineered cells back into peripheral nerve to slow disease progression.

**Statement of Benefit to California:** The proposed research will benefit the State of California as it will support the career development of a uniquely trained physician scientist to establish an innovative translational stem cell research program aimed toward direct clinical application to patients. The cutting edge technologies proposed are directly in line with the fundamental purpose of the California Initiative for Regenerative Medicine. If successful, both scientific and patient advocate organizations would recognize that these advances came directly from the unique efforts of CIRM and the State of California to lead the world in stem cell research. Finally, as a result of funding of this award, further financial investments from private and public funding organizations would directly benefit the State in the years to come.

---

**Source URL:** <https://www.cirm.ca.gov/our-progress/awards/human-ipsc-modeling-and-therapeutics-degenerative-peripheral-nerve-disease>